ABSTRACT

The present invention provides a series of novel dystrophin minigenes that retain the essential biological functions. The expression of the dystrophin minigenes may be controlled by a regulatory element along with a small polyadenylation signal. The entire gene expression cassettes may be readily packaged into a viral vector, preferably an AAV vector. The present invention further defines the minimal functional domains of dystrophin and provides ways to optimize and create new versions of dystrophin minigenes. Finally, the present invention provides a method of treatment for Duchenne muscular dystrophy (DMD) and Becker muscular dystrophy (BMD).